
Full Capsid
Partial Capsid
Empty Capsid

FIG. 1B

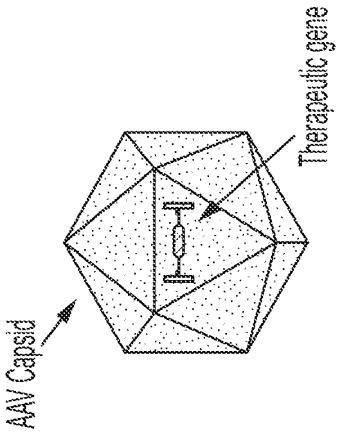

Therapeutic gene
AAV Capsid

FIG. 1A

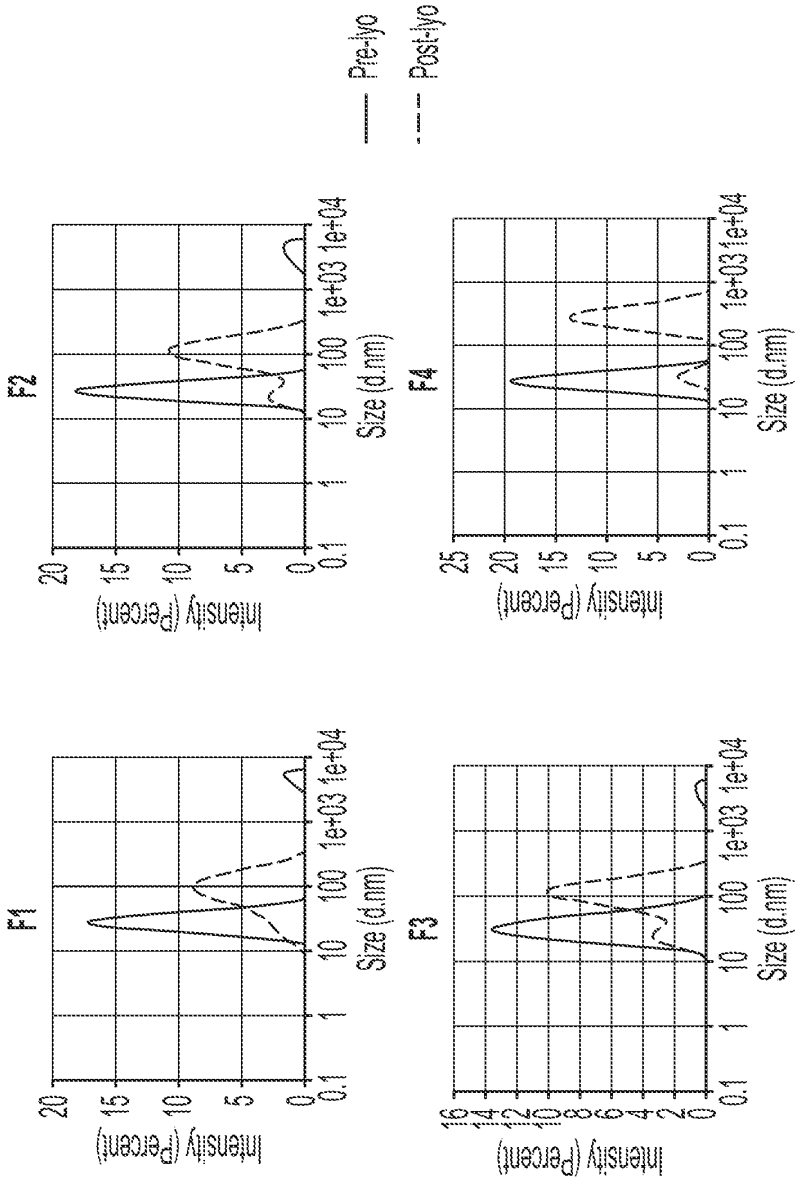

Pre-lyo
Post-lyo
F1
F2
F3
F4
Intensity (Percent)
Size (d.nm)
0.1
1
10
100
1e+03
1e+04

FIG. 2

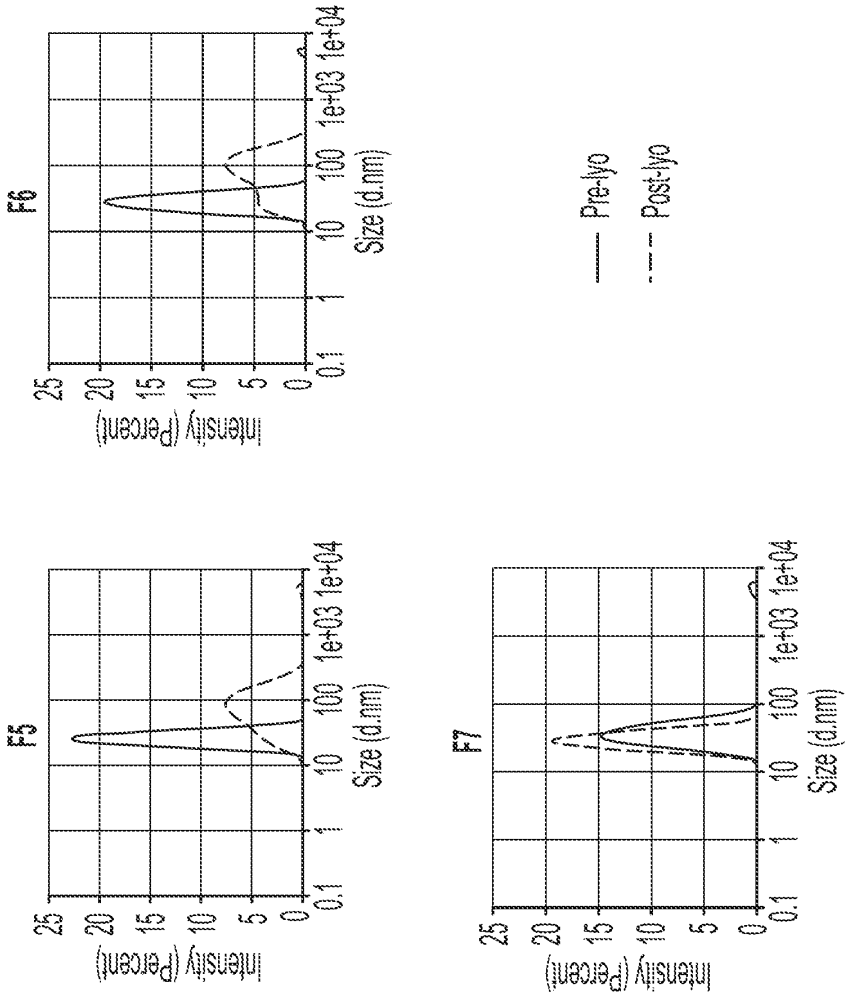


FIG. 2
CONTINUED

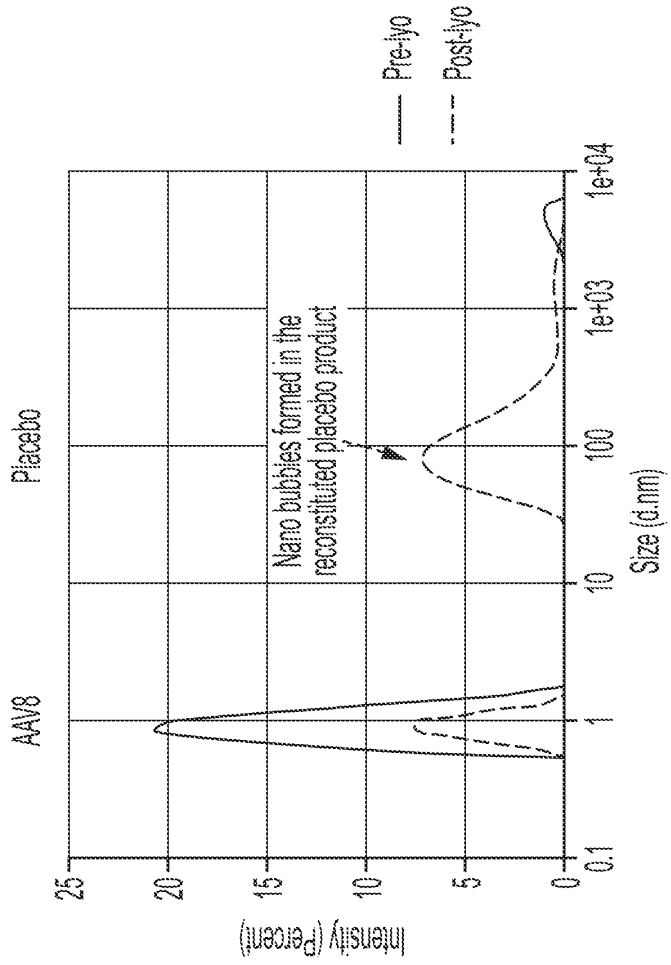

Pre-lyo
Post-lyo
Placebo
AAV8
Nano bubbles formed in the reconstituted placebo product
Intensity (Percent)
Size (d.nm)
25
20
15
10
5
0
0.1
1
10
100
1e+03
1e+04

FIG. 3

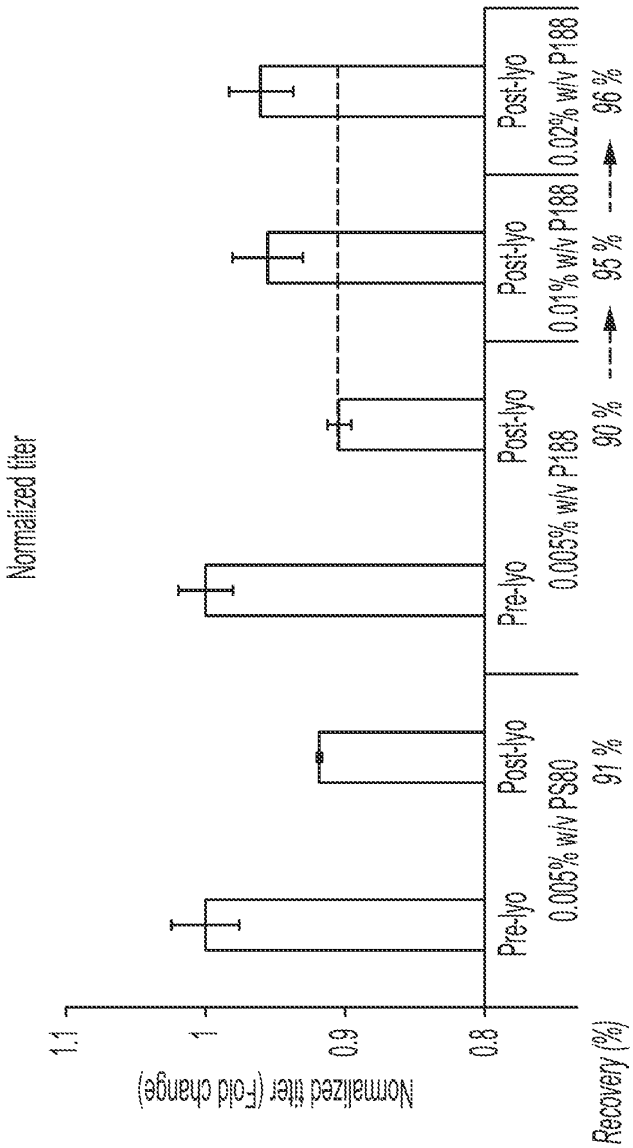

Normalized titer
Normalized titer (Fold change)
1.1
1
0.9
0.8
Pre-lyo
Post-lyo
0.005% w/v PS80
91 %
Pre-lyo
Post-lyo
0.005% w/v P188
90 %
Post-lyo
0.01% w/v P188
95 %
Post-lyo
0.02% w/v P188
96 %
Recovery (%)

FIG. 4

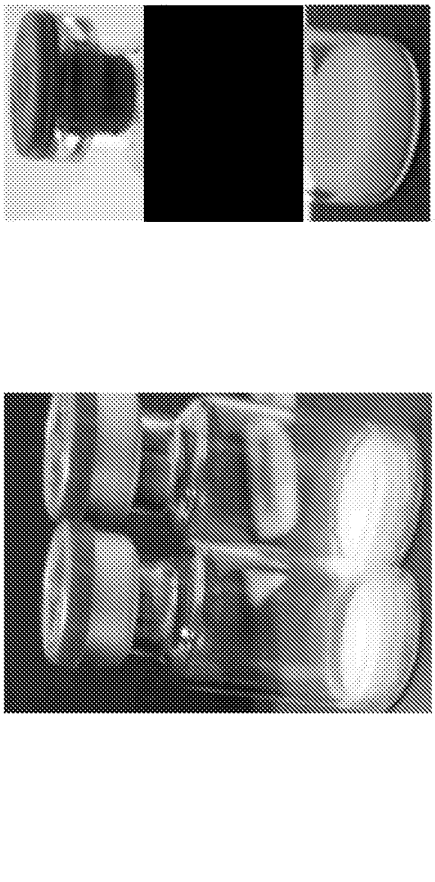


FIG. 5

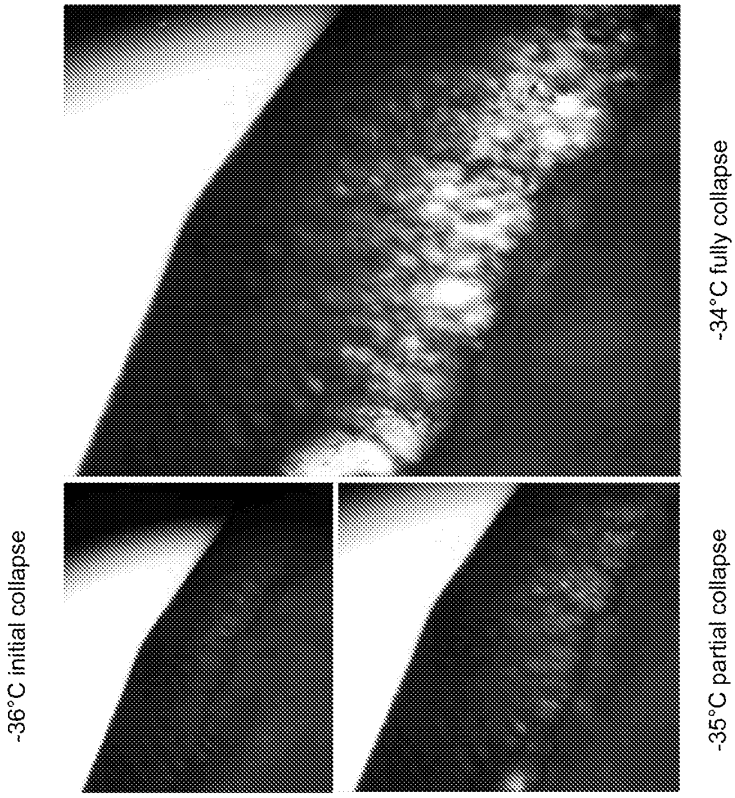

-34°C fully collapse
-36°C initial collapse
-35°C partial collapse

FIG. 6A

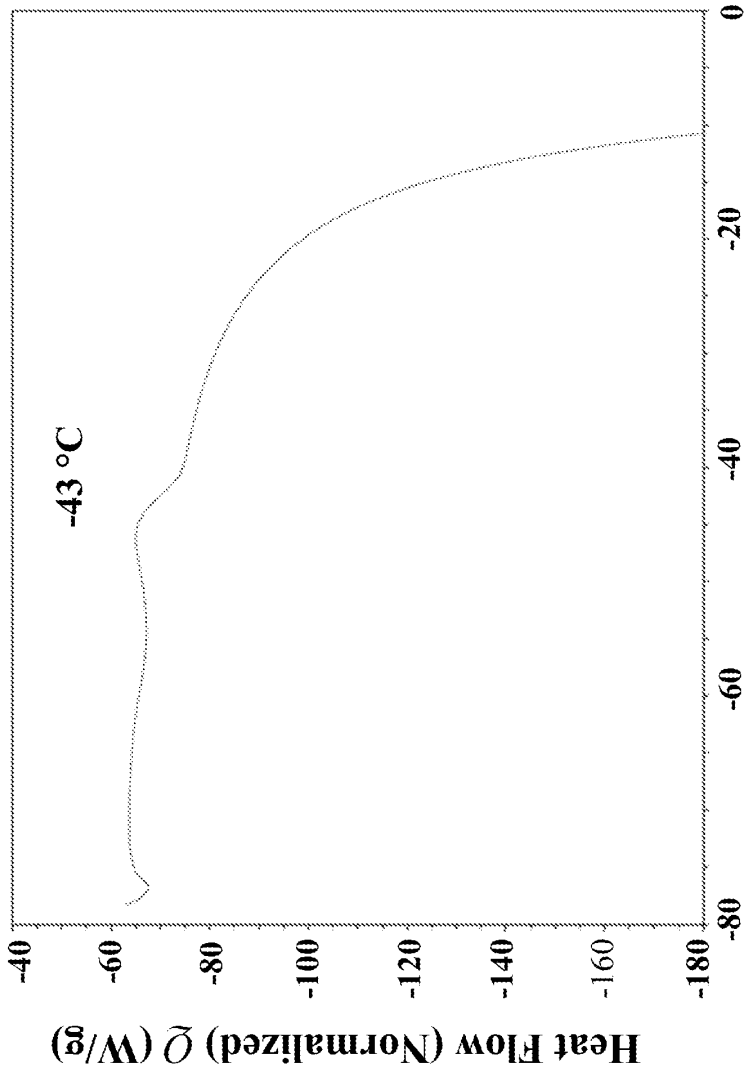

-43 °C
-40
-60
-80
-100
-120
-140
-160
-180
0
-20
-40
-60
-80
Heat Flow (Normalized) Q (W/g)
Temperature T (°C)

FIG. 6B

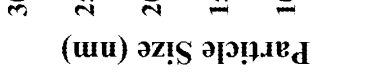


FIG. 7A

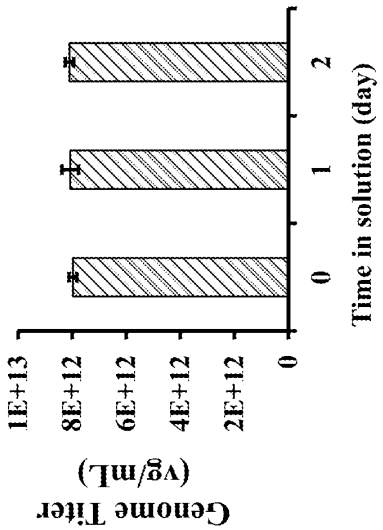


FIG. 7B

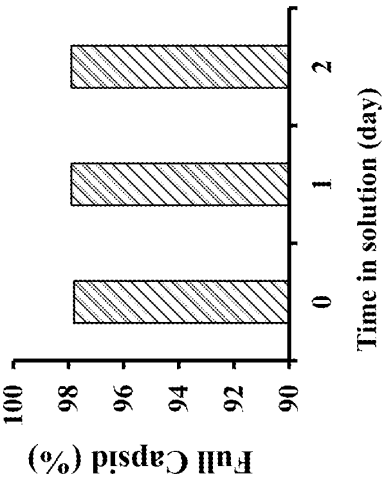


FIG. 7C

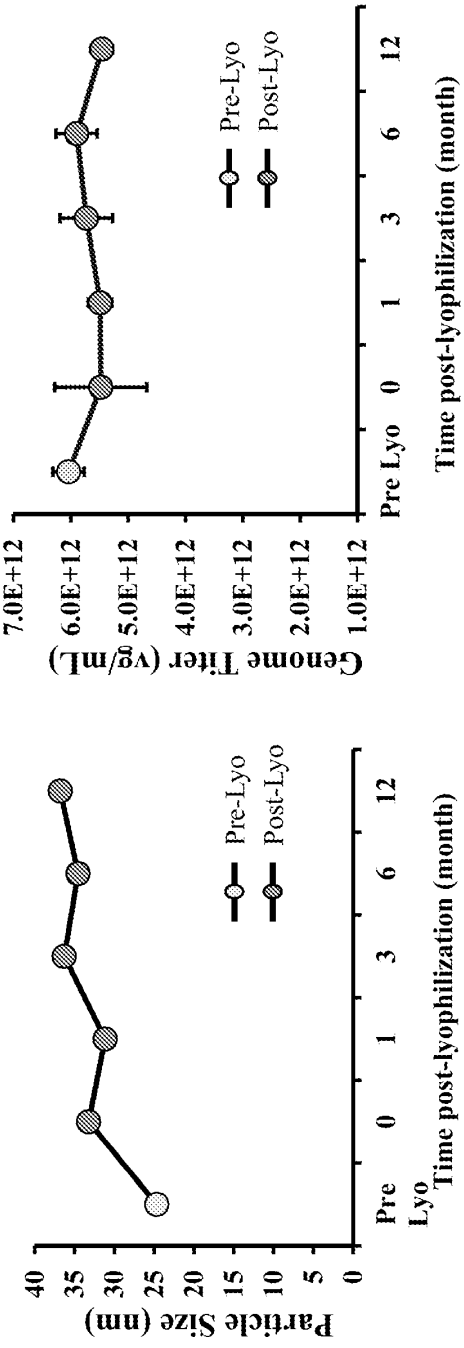


FIG. 8A

FIG. 8B

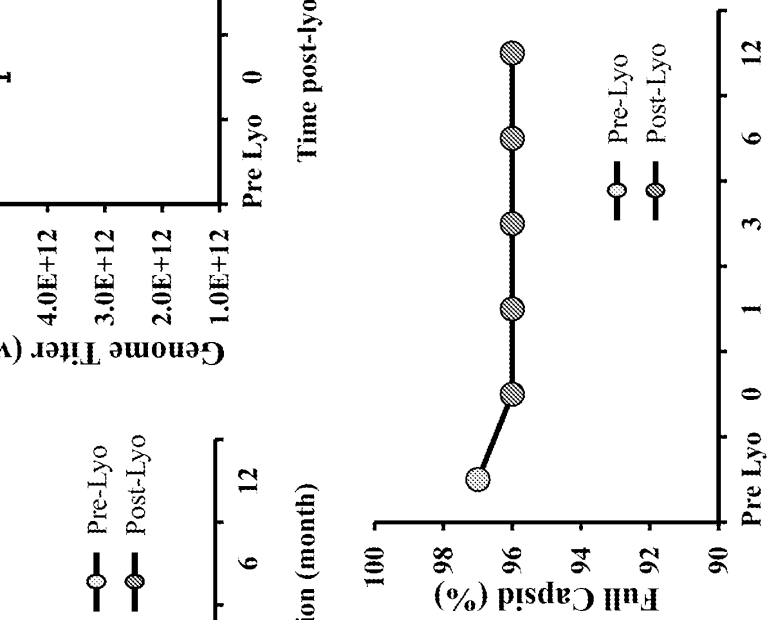


FIG. 8C

Formulation	Base Formulation	NaCl	L-Arg HCl	$MgCl_2$
Optimized Formulation (control)	10 mM Tris, 5% sucrose, 3.3% mannitol, 0.01% P188, pH 7.3	30mM	**80mM**	0
F8		80 mM	**0**	0
F9		40 mM	**40 mM**	0
F10		80 mM	**0**	2 mM

FIG. 9A

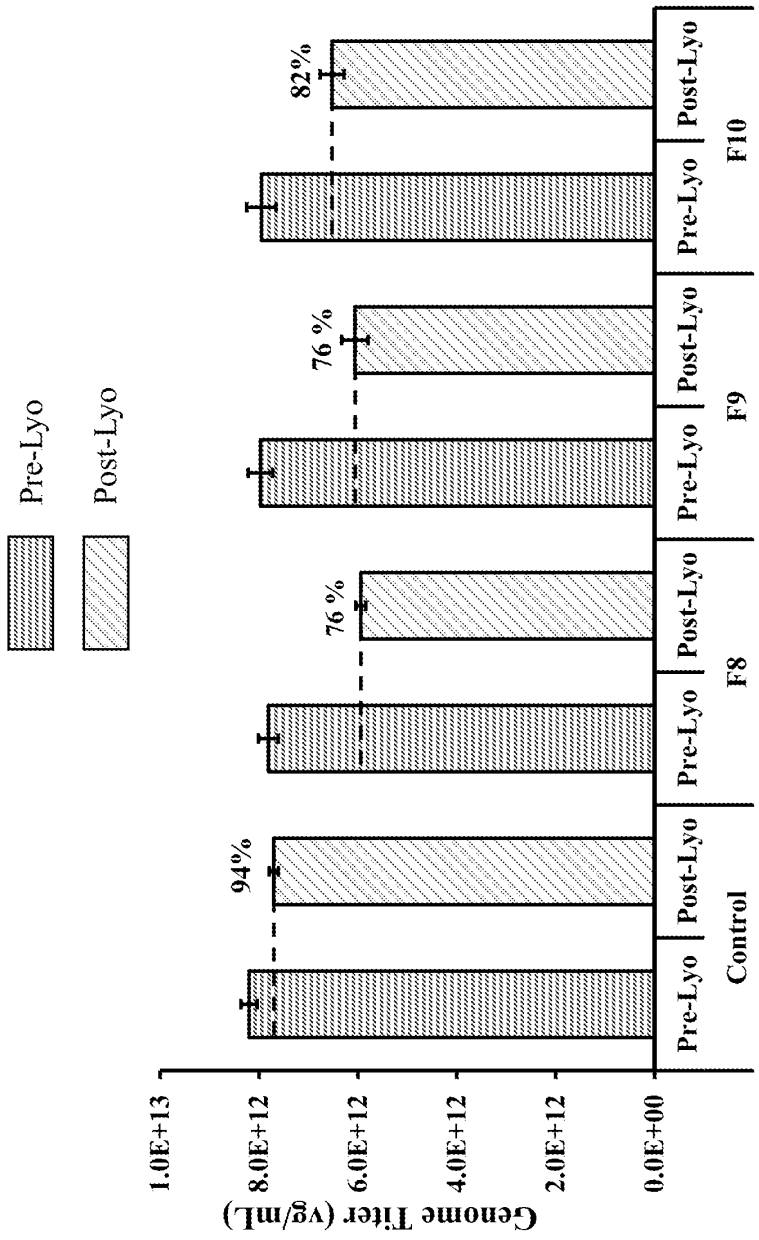

Pre-Lyo
Post-Lyo
Genome Titer (vg/mL)
1.0E+13
8.0E+12
6.0E+12
4.0E+12
2.0E+12
0.0E+00
94%
76 %
76 %
82%
Pre-Lyo
Post-Lyo
Control
Pre-Lyo
Post-Lyo
F8
Pre-Lyo
Post-Lyo
F9
Pre-Lyo
Post-Lyo
F10

FIG. 9B

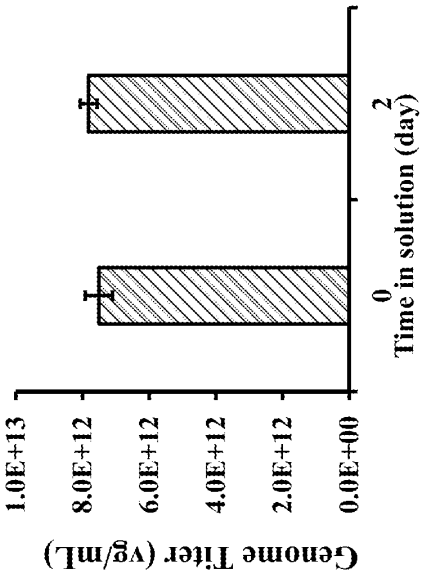


FIG. 10B

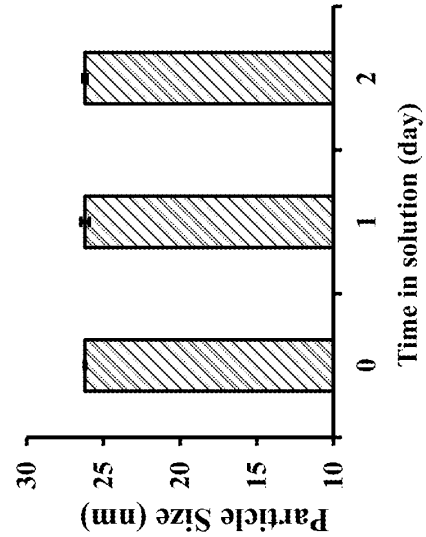


FIG. 10A

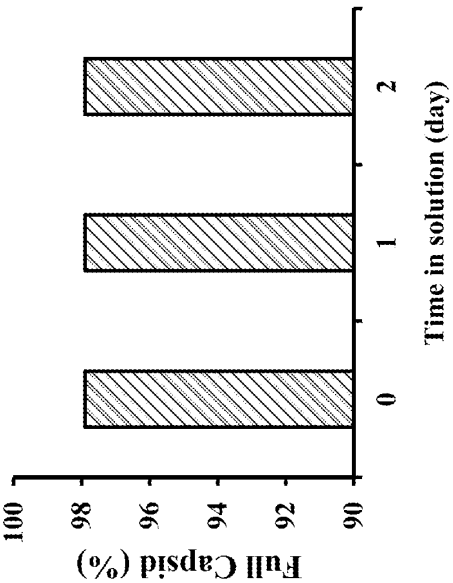


FIG. 10C

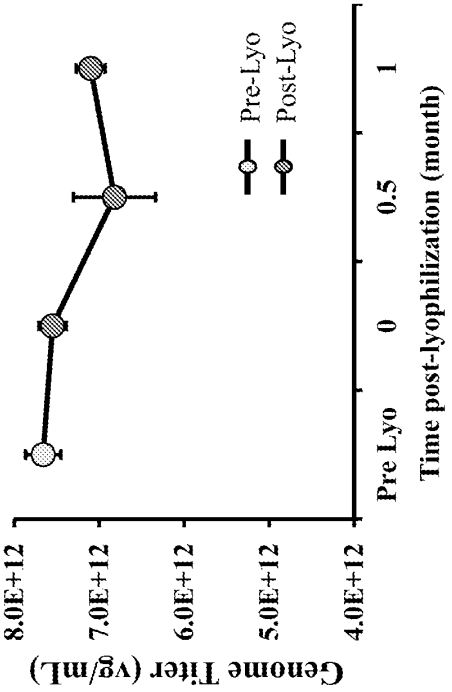


FIG. 11B

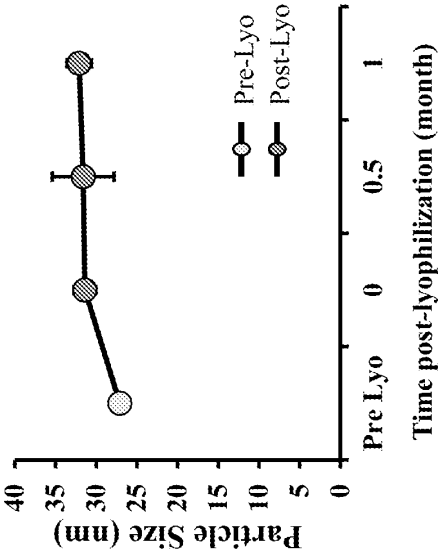


FIG. 11A

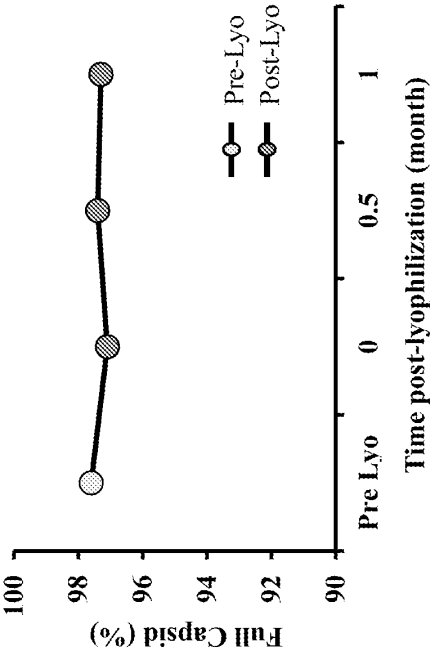


FIG. 11C